## Phenylketonuria (PKU) (mRNA-3283)

**Last program update: August 5, 2020**

<table>
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<th>Modality</th>
<th>ID #</th>
<th>Program Indication</th>
<th>Preclinical development</th>
<th>Phase 1</th>
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<td>mRNA-3704</td>
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**mRNA-3283 is in IND-enabling GLP toxicology studies**
Phenylketonuria (PKU) overview

Opportunity to address unmet need

• PKU is a rare inherited metabolic disease resulting from a deficiency in the metabolism of phenylalanine, or PHE, due to mutations within the enzyme phenylalanine hydroxylase, PAH

• Disease burden: Progressive mental disability from tyrosine deprivation

• Target population: Incidence of ~1:10K-15K live births in the US
  — ~21,000-32,000 patients in the US

• Approved therapies include:
  — 20-56% of patients respond favorably to BioMarin’s Kuvan
  — Non responsive patients treated with restricted diet
  — Biomarin’s Palynziq was approved in May 2018

Modernat concept: IV-administered mRNA encoding PAH enzyme to restore deficient or defective intracellular enzyme activity
Phenylketonuria (PKU) (mRNA-3283)
Preclinical data – restoration of enzymatic activity

Study Design:

- Species: Mouse
- Animals: PAH-/
- Dose: 0.5 mpk
- Dosing Schedule: weekly
- Injection Route: IV
- Sample Size: [5-6]

We have demonstrated the ability to impact PHE levels with repeat dosing of our mRNA in pre-clinical studies

*Data not collected due to a snow storm
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